

SEQUENCE LISTING

<110> CANFIELD, William
KORNFELD, Stuart

<120> EXPRESSION OF LYSOSOMAL HYDROLASE IN CELLS EXPRESSING PRO-N-
ACETYLGLUCOSAMINE-1-PHOSPHODIESTER ALPHA-N-ACETYL GLUCOSIMANIDASE

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<170> PatentIn version 3.1

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102234122101

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Tyr His Val Leu Phe Asp Ser Tyr Arg Asp Asn Ile Ala Gly Lys Ser
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Phe Gln Asn Arg Leu Cys Leu Pro Met Pro Ile Asp Val Val Tyr Thr
 65 70 75 80

Trp Val Asn Gly Thr Asp Leu Glu Leu Leu Lys Glu Leu Gln Gln Val
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Arg Glu Gln Met Glu Glu Glu Gln Lys Ala Met Arg Glu Ile Leu Gly
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Lys Asn Thr Thr Glu Pro Thr Lys Lys Ser Glu Lys Gln Leu Glu Cys
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Leu Leu Thr His Cys Ile Lys Val Pro Met Leu Val Leu Asp Pro Ala
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Leu Pro Ala Asn Ile Thr Leu Lys Asp Val Pro Ser Leu Tyr Pro Ser
145 150 155 160

Phe His Ser Ala Ser Asp Ile Phe Asn Val Ala Lys Pro Lys Asn Pro
165 170 175

Ser Thr Asn Val Ser Val Val Val Phe Asp Ser Thr Lys Asp Val Glu
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Asp Ala His Ser Gly Leu Leu Lys Gly Asn Ser Arg Gln Thr Val Trp
195 200 205

Arg Gly Tyr Leu Thr Thr Asp Lys Glu Val Pro Gly Leu Val Leu Met
210 215 220

Gln Asp Leu Ala Phe Leu Ser Gly Phe Pro Pro Thr Phe Lys Glu Thr
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Asn Gln Leu Lys Thr Lys Leu Pro Glu Asn Leu Ser Ser Lys Val Lys
245 250 255

Leu Leu Gln Leu Tyr Ser Glu Ala Ser Val Ala Leu Leu Lys Leu Asn
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Asn Pro Lys Asp Phe Gln Glu Leu Asn Lys Gln Thr Lys Lys Asn Met
275 280 285

Thr Ile Asp Gly Lys Glu Leu Thr Ile Ser Pro Ala Tyr Leu Leu Trp
290 295 300

Asp Leu Ser Ala Ile Ser Gln Ser Lys Gln Asp Glu Asp Ile Ser Ala
305 310 315 320

Ser Arg Phe Glu Asp Asn Glu Glu Leu Arg Tyr Ser Leu Arg Ser Ile
325 330 335

Glu Arg His Ala Pro Trp Val Arg Asn Ile Phe Ile Val Thr Asn Gly
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805

810

815

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Glu Lys Pro Pro Ser Leu Ile Val Pro Leu Glu Ser Gln Met Thr Lys
835 840 845

Glu Lys Lys Ile Thr Gly Lys Glu Lys Glu Asn Ser Arg Met Glu Glu
850 855 860

Asn Ala Glu Asn His Ile Gly Val Thr Glu Val Leu Leu Gly Arg Lys
865 870 875 880

Leu Gln His Tyr Thr Asp Ser Tyr Leu Gly Phe Leu Pro Trp Glu Lys
885 890 895

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Phe Asp Lys Thr Ser Phe His Lys Val Arg His Ser Glu Asp Met Gln
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Phe Ala Phe Ser Tyr Phe Tyr Tyr Leu Met Ser Ala Val Gln Pro Leu
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Ala Ser Pro Asn Arg Ile Arg Val
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REPORT

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Phe Arg Leu Ser Gly Lys Cys Phe Ser Leu Val Glu Ser Thr Tyr Lys
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Arg Trp Asn Ala Tyr Ser Gly Ile Leu Gly Ile Trp His Glu Trp Glu
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Cys Arg Ser Arg Ser Arg Gln Ser Lys Val Glu Leu Ala Cys Gly Lys
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Ser Asn Arg Leu Ala His Val Ser Glu Pro Ser Thr Cys Val Tyr Ala
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Tyr Pro Thr Leu Pro Glu Ala Leu Gln Arg Gln Trp Asp Gln Val Glu
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Gln Asp Leu Ala Asp Glu Leu Ile Thr Pro Gln Gly His Glu Lys Leu
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Leu Arg Thr Leu Phe Glu Asp Ala Gly Tyr Leu Lys Thr Pro Glu Glu
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Thr Leu Glu Asn Cys Arg Lys Ala His Lys Glu Leu Ser Lys Glu Ile
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Lys Arg Leu Lys Gly Leu Leu Thr Gln His Gly Ile Pro Tyr Thr Arg
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Ala Ser Arg Phe Glu Asp Asn Glu Glu Leu Arg Tyr Ser Leu Arg Ser 325 330 335		
Ile Glu Arg His Ala Pro Trp Val Arg Asn Ile Phe Ile Val Thr Asn 340 345 350		
Gly Gln Ile Pro Ser Trp Leu Asn Leu Asp Asn Pro Arg Val Thr Ile 355 360 365		
Val Thr His Gln Asp Ile Phe Gln Asn Leu Ser His Leu Pro Thr Phe 370 375 380		
Ser Ser Pro Ala Ile Glu Ser His Ile His Arg Ile Glu Gly Leu Ser 385 390 395 400		
Gln Lys Phe Ile Tyr Leu Asn Asp Asp Val Met Phe Gly Lys Asp Val 405 410 415		
Trp Pro Asp Asp Phe Tyr Ser His Ser Lys Gly Gln Lys Val Tyr Leu 420 425 430		
Thr Trp Pro Val Pro Asn Cys Ala Glu Gly Cys Pro Gly Ser Trp Ile 435 440 445		
Lys Asp Gly Tyr Cys Asp Lys Ala Cys Asn Thr Ser Pro Cys Asp Trp 450 455 460		
Asp Gly Gly Asn Cys Ser Gly Asn Thr Ala Gly Asn Arg Phe Val Ala 465 470 475 480		
Arg Gly Gly Gly Thr Gly Asn Ile Gly Ala Gly Gln His Trp Gln Phe 485 490 495		

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Tyr Lys Val Thr Leu Leu Pro Asn Gln Thr His Tyr Val Val Pro Lys
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Gly Glu Tyr Leu Ser Tyr Phe Ser Phe Ala Asn Ile Ala Arg Lys Arg
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Ile Glu Gly Thr Tyr Ser Asp Asn Pro Ile Ile Arg His Ala Ser Ile
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Ala Asn Lys Trp Lys Thr Leu His Leu Ile Met Pro Gly Gly Met Asn
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Val Thr Pro Leu Pro Gln Ala Asp Val Pro Phe Glu Asp Val Pro Lys
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Arg Gly Asp Ile Thr Leu Lys Gly Tyr Asn Leu Ser Lys Ser Ala Leu
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Leu Arg Ser Phe Leu Gly Asn Ser Leu Asp Thr Lys Ile Lys Pro Gln
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Ala Arg Thr Asp Glu Thr Lys Gly Asn Leu Glu Val Pro Gln Glu Asn
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Pro Ser His Arg Arg Pro His Gly Phe Ala Gly Glu His Arg Ser Glu
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820 825 830

Val Pro Pro Glu Ser His Leu Pro Lys Glu Glu Glu Ser Asp Arg Ala
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Gln Gln Asn Tyr Pro Gly Phe Leu Pro Trp Glu Lys Lys Lys Tyr Phe
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Pro	Leu 115	Ser	Leu	Gln	Asp	Leu	Thr 120	Gly	Leu	Glu	His	Met 125	Leu	Ile	Asn	
Cys	Ser 130	Lys	Met	Leu	Pro	Ala 135	Asn	Ile	Thr	Gln	Leu 140	Asn	Asn	Ile	Pro	
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Pro Arg Glu Tyr Arg Asn Arg Phe Leu His Met His Glu Leu Gln Glu
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Trp Arg Ala Tyr Arg Asp Lys Leu Lys Phe Trp Thr His Cys Val Leu
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Ala Thr Leu Ile Ile Phe Thr Ile Phe Ser Phe Phe Ala Glu Gln Ile
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Pro Asn Thr Phe Gly Leu Asn Asn Pro Phe Leu Pro Gln Ala Ser Arg
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Leu Gln Pro Lys Arg Glu Pro Ser Ala Val Ser Gly Pro Leu His Leu
50 55 60

Phe Arg Leu Ala Gly Lys Cys Phe Ser Leu Val Glu Ser Thr Tyr Lys
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Tyr Glu Phe Cys Pro Phe His Asn Val Thr Gln His Glu Gln Thr Phe
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Arg Trp Asn Ala Tyr Ser Gly Ile Leu Gly Ile Trp His Glu Trp Glu
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Ile Ile Asn Asn Thr Phe Lys Gly Met Trp Met Thr Asp Gly Asp Ser
115 120 125

Cys His Ser Arg Ser Arg Gln Ser Lys Val Glu Leu Thr Cys Gly Lys
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Ile Asn Arg Leu Ala His Val Ser Glu Pro Ser Thr Cys Val Tyr Ala
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Leu Thr Phe Glu Thr Pro Leu Val Cys His Pro His Ser Leu Leu Val
165 170 175

Tyr Pro Thr Leu Ser Glu Ala Leu Gln Gln Arg Leu Asp Gln Val Glu
180 185 190

Gln Asp Leu Ala Asp Glu Leu Ile Thr Pro Gln Gly Tyr Glu Lys Leu
195 200 205

Leu Arg Val Leu Phe Glu Asp Ala Gly Tyr Leu Lys Val Pro Gly Glu
210 215 220

Glu Asn Leu Ser Ser Lys Ile Lys Leu Leu Gln Leu Tyr Ser Glu Ala
 20 25 30

Ser Val Ala Leu Leu Lys Leu Asn Asn Pro Lys Gly Phe Pro Glu Leu
 35 40 45

Asn Lys Gln Thr Lys Lys Asn Met Ser Ile Ser Gly Lys Glu Leu Ala
 50 55 60

Ile Ser Pro Ala Tyr Leu Leu Trp Asp Leu Ser Ala Ile Ser Gln Ser
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Lys Gln Asp Glu Asp Val Ser Ala Ser Arg Phe Glu Asp Asn Glu Glu
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Leu Arg Tyr Ser Leu Arg Ser Ile Glu Arg His Asp Ser Met Ser Pro
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Asn Arg Ala Tyr Gly Phe Lys Ala Arg His Val Leu Ala His Val Gly 275 280 285		
Phe Leu Ile Asp Lys Asp Ile Val Glu Ala Met Gln Arg Arg Phe His 290 295 300		
Gln Gln Ile Leu Asp Thr Ala His Gln Arg Phe Arg Ala Pro Thr Asp 305 310 315 320		
Leu Gln Tyr Ala Phe Ala Tyr Tyr Ser Phe Leu Met Ser Glu Thr Lys 325 330 335		
Val Met Ser Val Glu Glu Ile Phe Asp Glu Phe Asp Thr Asp Gly Ser 340 345 350		
Ala Thr Trp Ser Asp Arg Glu Val Arg Thr Phe Leu Thr Arg Ile Tyr 355 360 365		
Gln Pro Pro Leu Asp Trp Ser Ala Met Arg Tyr Phe Glu Glu Val Val 370 375 380		
Gln Asn Cys Thr Arg Asn Leu Gly Met His Leu Lys Val Asp Thr Val 385 390 395 400		
Glu His Ser Thr Leu Val Tyr Glu Arg Tyr Glu Asp Ser Asn Leu Pro 405 410 415		
Thr Ile Thr Arg Asp Leu Val Val Arg Cys Pro Leu Leu Ala Glu Ala 420 425 430		
Leu Ala Ala Asn Phe Ala Val Arg Pro Lys Tyr Asn Phe His Val Ser 435 440 445		

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Asp Asp Asp Leu Leu Leu Pro Tyr Pro Arg Ala Arg Ala Arg Leu Pro
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Arg Asp Cys Thr Arg Val Arg Ala Gly Asn Arg Glu His Glu Ser Trp
50 55 60

Pro Pro Pro Pro Ala Thr Pro Gly Ala Gly Gly Leu Ala Val Arg Thr
65 70 75 80

Phe Val Ser His Phe Arg Asp Arg Ala Val Ala Gly His Leu Thr Arg
85 90 95

Ala Val Glu Pro Leu Arg Thr Phe Ser Val Leu Glu Pro Gly Gly Pro
100 105 110

Gly Gly Cys Ala Ala Arg Arg Arg Ala Thr Val Glu Glu Thr Ala Arg
115 120 125

Ala Ala Asp Cys Arg Val Ala Gln Asn Gly Gly Phe Phe Arg Met Asn
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Ser Gly Glu Cys Leu Gly Asn Val Val Ser Asp Glu Arg Arg Val Ser
145 150 155 160

Ser Ser Gly Gly Leu Gln Asn Ala Gln Phe Gly Ile Arg Arg Asp Gly
165 170 175

Thr Leu Val Thr Gly Tyr Leu Ser Glu Glu Glu Val Leu Asp Thr Glu
180 185 190

Asn Pro Phe Val Gln Leu Leu Ser Gly Val Val Trp Leu Ile Arg Asn
195 200 205

Gly Ser Ile Tyr Ile Asn Glu Ser Gln Ala Thr Glu Cys Asp Glu Thr
210 215 220

Gln Glu Thr Gly Ser Phe Ser Lys Phe Val Asn Val Ile Ser Ala Arg
225 230 235 240

Thr Ala Ile Gly His Asp Arg Lys Gly Gln Leu Val Leu Phe His Ala
245 250 255

Asp Gly His Thr Glu Gln Arg Gly Ile Asn Leu Trp Glu Met Ala Glu

270

Arg Leu His Gly Asp Tyr Ala Tyr His Pro Leu Gln Glu Met Asn Gly
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Glu Pro Leu Ala Ala Glu Lys Glu Gln Pro Gly Gly Ala His Asn Pro
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Phe Lys Asp
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 <213> Mus musculus

<400> 20

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Arg Pro Ser Arg Asp Cys Ala Arg Val Arg Ser Gly Ser Pro Glu Gln
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Glu Ser Trp Pro Pro Pro Pro Leu Ala Thr His Glu Pro Arg Ala Pro
 35 40 45

Ser His His Ala Ala Val Arg Thr Phe Val Ser His Phe Glu Gly Arg
 50 55 60

Ala Val Ala Gly His Leu Thr Arg Val Ala Asp Pro Leu Arg Thr Phe
 65 70 75 80

Ser Val Leu Glu Pro Gly Gly Ala Gly Gly Cys Gly Gly Arg Ser Ala
 85 90 95

Ala Ala Thr Val Glu Asp Thr Ala Val Arg Ala Gly Cys Arg Ile Ala
100 105 110

Gln Asn Gly Gly Phe Phe Arg Met Ser Thr Gly Glu Cys Leu Gly Asn
115 120 125

Val Val Ser Asp Gly Arg Leu Val Ser Ser Ser Gly Gly Leu Gln Asn
130 135 140

Ala Gln Phe Gly Ile Arg Arg Asp Gly Thr Ile Val Thr Gly Ser Cys
145 150 155 160

Leu Glu Glu Glu Val Leu Asp Pro Val Asn Pro Phe Val Gln Leu Leu
165 170 175

Ser Gly Val Val Trp Leu Ile Arg Asn Gly Asn Ile Tyr Ile Asn Glu
180 185 190

Ser Gln Ala Ile Glu Cys Asp Glu Thr Gln Glu Thr Gly Ser Phe Ser
195 200 205

Lys Phe Val Asn Val Met Ser Ala Arg Thr Ala Val Gly His Asp Arg
210 215 220

Glu Gly Gln Leu Ile Leu Phe His Ala Asp Gly Gln Thr Glu Gln Arg
225 230 235 240

Gly Leu Asn Leu Trp Glu Met Ala Glu Phe Leu Arg Gln Gln Asp Val
245 250 255

Val Asn Ala Ile Asn Leu Asp Gly Gly Gly Ser Ala Thr Phe Val Leu
260 265 270

Asn Gly Thr Leu Ala Ser Tyr Pro Ser Asp His Cys Gln Asp Asn Met
275 280 285

Trp Arg Cys Pro Arg Gln Val Ser Thr Val Val Cys Val His Glu Pro
290 295 300

Arg Cys Gln Pro Pro Asp Cys Ser Gly His Gly Thr Cys Val Asp Gly
305 310 315 320

His Cys Glu Cys Thr Ser His Phe Trp Arg Gly Glu Ala Cys Ser Glu
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Leu Asp Cys Gly Pro Ser Asn Cys Ser Gln His Gly Leu Cys Thr Ala
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Gly Cys His Cys Asp Ala Gly Trp Thr Gly Ser Asn Cys Ser Glu Glu
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Cys Pro Leu Gly Trp Tyr Gly Pro Gly Cys Gln Arg Pro Cys Gln Cys
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Glu His Gln Cys Phe Cys Asp Pro Gln Thr Gly Asn Cys Ser Ile Ser
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Gln Val Arg Gln Cys Leu Gln Pro Thr Glu Ala Thr Pro Arg Ala Gly
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Glu Leu Ala Ser Phe Thr Arg Thr Thr Trp Leu Ala Leu Thr Leu Thr
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Leu Ile Phe Leu Leu Leu Ile Ser Thr Gly Val Asn Val Ser Leu Phe
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Leu Gly Ser Arg Ala Glu Arg Asn Arg His Leu Asp Gly Asp Tyr Val
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<211> 9792

<212> DNA

<213> Mus musculus

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